Welcome!

Outcome Measures and Infrastructure for Phase III Studies in Batten Disease (JNCL)

December 6-7, 2013 Rochester, NY USA

Disclosures and Acknowledgments





- Dan Parr, Magda Ramzy, Alyssa Thatcher, Sara Defendorf (Admin. Support)
- All participants, from near and far!
- Parents Lori Sikorra (California, USA) & Ellen Bletsoe (Thrapston, UK)
- Advocacy Groups BDSRA (Chris Leonard) & BBDF (Lisa Beth Furstenberg)

Acknowledgments - A Strong Foundation

International Conference on NCL

12th Hamburg, Germany (2009) 13th London, England (2012)

14^{th.} Córdoba, Argentina (2014)

First International Education Conference on Batten Disease Örebro, Sweden (2006)

Drug Discovery in JNCL Conf.Beyond Batten (2011)

Neurobiology of Disease Symposium Child Neurology Society meeting South Beach, CA, USA (2012)

NCL (National Contest for Life) Congress.

Annual, Hamburg, Germany

NOT DESCRIBED. A CATEMAX COR DATTER PARTY OF

http://www.ucl.ac.uk/ncl/meetingspast.shtml

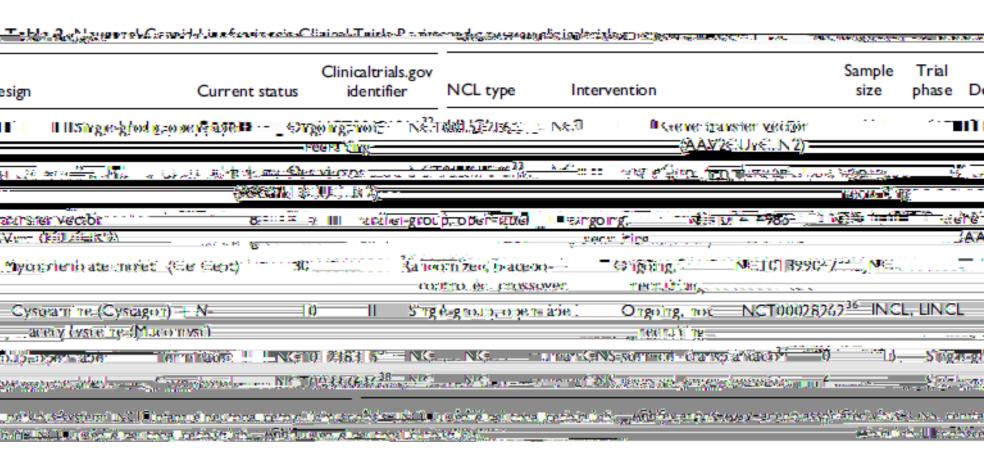
1980: International Symposium on Human and Animal Models of Ceroid-lipofuscinosis, Røros, Norway

1st
Worldwide
Meeting of
Batten
Disease
International
Alliance
(BDIA)



Background: Juvenile Neuronal Ceroid Lipofuscinosis (CLN3)

Background



Augustine et al. Clinical Trials in Rare Disease: Challenges and Opportunities. JCN 2013, 28: 1142

Background:

 "need to develop new tools to more rapidly assess the effects of potential therapies" 1

• Sensitive, responsive, "well-defined and reliable <u>endpoint</u> <u>measures</u> [are] particularly important in rare diseases where sample sizes are limited" ², in order to assess a clinically meaningful change in response to intervention.

Meeting Objectives

To bring together clinical research experts in JNCL and rare diseases to focus on establishing common ground for outcomes and infrastructure in support of

Meeting Structure: Day 1 Formal Talks

THEMES

Background on the NCLs

Rare disease clinical research Experimental therapeutics, Clinical trial endpoints

Challenging ourselves...

Registries, Clinical endpoints, What matters to parents

State of the Science in JNCL, with focus on clinical trial applications

Poster Presentations



Day 2 Working Groups

Working Group: Session 1

Propose endpoints / registry activities

Consider: reliable, valid, relevant, feasible Large Group Discussion: Session 1

SUMMARY

&

FEEDBACK

Working Group: Session 2

Revise proposed endpoints / registry activities

Draft work plan

Large Group Discussion: Session 2

FINAL SUMMARY

Action I tems

Meeting Outcomes

1. A set of potential clinical endpoints in JNCL and a plan for their development and validation for future Phase III studies.

2. A plan for expansion of NCL Patient Registries, to incorporate clinical trial endpoint work.

- 3. Enduring and evolving content
- Manuscript of proceedings
- Website (with PDFs of Day 1 talks and reference materials to support experimental therapeutics and Phase III trials in JNCL. We hope these materials will be useful for clinical trials in the other NCLs as well.
- Maintain interactions among participants at future meetings

References

- 1. Strimbu K & Tavel JA. What are biomarkers? Curr Opin HIV AIDS 2010. 5(8) 463-466
- 2. Gliklich RE, Dreyer NA, editors. Registries for Evaluating Patient Outcomes: A User's Guide. 2nd edition. Rockville (MD): Agency for Healthcare Research and Quality (US); 2010 Sep. Available from: http://www.ncbi.nlm.nih.gov/books/NBK49444/
- 3. McDowell I. Measuring Health: A guide to rating scales and questionnaires, Third Edition. 2006. Oxford Univ. Press.
- 4. Robert Temple, FDA; Biomarkers Definitions Working Group 2001; Institute of Medicine 2010